Transplantation of CRISPR-CAS9 Corrected Hematopoietic Stem Cells (CRISPR_SCD001) in Patients with Severe Sickle Cell Disease

Grant Award Details

Transplantation of CRISPR-CAS9 Corrected Hematopoietic Stem Cells (CRISPR_SCD001) in Patients with Severe Sickle Cell Disease

Grant Type: Cure Sickle Cell Initiative Clinical Trial Stage Projects

Grant Number: CLIN2SCD-11722

Investigator:

Name:	Mark Walters
Institution:	University of California, San Francisco
Type:	PI

Disease Focus: Blood Disorders, Sickle Cell Disease

Human Stem Cell Use: Adult Stem Cell

Award Value: $8,389,407

Status: Pre-Active

Grant Application Details

Application Title: Transplantation of CRISPR-CAS9 Corrected Hematopoietic Stem Cells (CRISPR_SCD001) in Patients with Severe Sickle Cell Disease

Public Abstract:

Therapeutic Candidate or Device

CRISPR corrected blood stem cells are manufactured from persons with severe sickle cell disease and returned by transplant to the same person

Indication

Persons with sickle cell disease (adults and adolescents) with repeated, severe painful and lung events are eligible for the clinical trial

Therapeutic Mechanism

Blood stem cells are harvested from blood in persons with severe sickle cell disease. The cells are treated with DNA editing tools (CRISPR) to convert the sickle-producing cells to healthy blood stem cells. If this sickle correction occurs in enough of the blood stem cells, it could help or even stop complications of sickle cell disease after the CRISPR treated stem cells are returned to the bloodstream. Persons receive chemotherapy before the re-infused, corrected cells set up in the marrow.

Unmet Medical Need

Sickle Cell Disease is inherited mostly by persons in under-represented minorities. It has not received enough attention and funding. CIRM is supporting research to discover new cures for sickle cell disease. This proposal could give many more persons with sickle cell disease a chance of cure.

Project Objective

Complete an early phase trial in up to 9 persons.

Major Proposed Activities

- Prepare to enroll patients at 2 clinical sites (UCSF and UCLA) by completing the protocol and readying the clinical and manufacturing teams.
- Enroll 3 adults with severe sickle cell disease, staggering the study schedule for a safety assessment before each new patient is treated.
- Enroll 3 more adults; if safety and clinical benefit are shown, enroll 3 adolescents on a staggered schedule. If promising, prepare for next study.

Statement of Benefit to California:

The economic and health burdens of sickle cell disease are significant. As many as 13,000 persons in California are affected. The benefit of a curative therapy such as CRISPR_SCD001 is that it has the potential to be available in all affected persons if its safety and effectiveness is proved. This benefit will be magnified if it can be delivered safely early in life, before the chronic life-long problems of sickle cell disease have occurred.

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